Human Neural Stem Cells (hNSCs) for neuroprotection in perinatal hypoxic-ischemic brain injury (HII)-Pre-IND-enabling Studies

Grant Award Details

Human Neural Stem Cells (hNSCs) for neuroprotection in perinatal hypoxic-ischemic brain injury (HII)-Pre-IND-enabling Studies

Grant Type: Therapeutic Translational Research Projects

Grant Number: TRAN1-11628

Investigator:

Name:	Evan Snyder
Institution:	Sanford-Burnham Medical Research Institute
Type:	PI

Disease Focus: Brain Injury, hypoxic, ischemic, Neurological Disorders, Pediatrics

Human Stem Cell Use: Adult Stem Cell

Award Value: $4,963,684

Status: Pre-Active

Grant Application Details

Application Title: Human Neural Stem Cells (hNSCs) for neuroprotection in perinatal hypoxic-ischemic brain injury (HII)-Pre-IND-enabling Studies

Public Abstract:

Translational Candidate

An established stable human neural stem cell line unmanipulated genetically & propagated under defined conditions

Area of Impact

Perinatal asphyxia (also called hypoxic-ischemic injury), a major untreatable cause of cerebral palsy & cognitive disability

Mechanism of Action

hNSCs rescue the penumbra, the part of the brain lesion following perinatal asphyxia that still has viable though endangered cells. Such rescue includes preserving tissue; host neuron growth; revascularization; inhibiting inflammation & scarring. Anatomic & behavioral improvement results. If strategically administered, hNSCs can supply their neuroprotective molecules in a manner that synergizes with standard-of-care, hypothermia, which is only marginally effective but must be offered to babies.

Unmet Medical Need

Perinatal hypoxic-ischemic brain injury is an untreatable common cause of CP & disability. Hypothermia (HT) is standard-of-care for this condition although it is only marginally-effective. Any new trial must include HT. We will coordinate hNSC administration to synergize with HT & improve outcome.

Project Objective

Pre-IND meeting, ultimately a Phase 1b/2a trial

Major Proposed Activities

- Ascertain the proper timing of hNSC administration in relation to hypothermia to achieve synergy
- Determine the manufacturing specifications & biodistribution of the hNSCs in anticipation of IND-enabling studies
- Preparation of a pre-IND package

Statement of Benefit to California:

Perinatal asphyxia occurs in 2-4/1000 births. Despite hyperthermia (which is only marginally effective), 80% of asphyxiated infants develop neurologic signs with 10-20% remaining significantly impaired (e.g., CP; disability; epilepsy). The cost to California economy is $1M/child in terms of lifelong medical & rehabilitative care; the impact on family dynamics is 2-5-fold greater than that. We believe stem cell-based interventions can improve these outcomes.

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